

(19) World Intellectual Property
Organization
International Bureau



(43) International Publication Date
20 January 2005 (20.01.2005)

PCT

(10) International Publication Number
WO 2005/005634 A2

- (51) International Patent Classification⁷: C12N 15/11
- (21) International Application Number: PCT/IT2004/000381
- (22) International Filing Date: 9 July 2004 (09.07.2004)
- (25) Filing Language: English
- (26) Publication Language: English
- (30) Priority Data:
RM2003A000335 9 July 2003 (09.07.2003) IT
- (71) Applicant (for all designated States except US): **UNIVERSITÀ DEGLI STUDI DI ROMA [IT/IT]**; La Sapienza, Piazzale A. Moro, 5, I-00185 Roma (IT).
- (72) Inventors; and
- (75) Inventors/Applicants (for US only): **BOZZONI, Irene** [IT/IT]; c/o Università degli Studi di Roma, La Sapienza, Piazzale A. Moro, 5, I-00185 Roma (IT). **DENTI, Michela, Alessandra** [IT/IT]; c/o Università degli Studi di Roma, La Sapienza, Piazzale A. Moro, 5, I-00185 Roma (IT). **ROSA, Alessandro** [IT/IT]; c/o Università degli Studi di Roma, La Sapienza, Piazzale A. Moro, 5, I-00185 Roma (IT).
- (74) Agents: **CAPASSO, Olga et al.**; De Simone & Partners S.p.A., Via Vincenzo Bellini, 20, I-00198 Roma (IT).
- (81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.
- (84) Designated States (unless otherwise indicated, for every kind of regional protection available): ARIPO (BW, GH, GM, KE, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).
- Published:
— without international search report and to be republished upon receipt of that report
- For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

(54) Title: siRNA EXPRESSION SYSTEM

(57) Abstract: The authors describe an expression system for the correct, stable and effective expression in mammalian cells of a siRNA comprising: a) a polymerase II RNA-dependent promoter derived from the U1 RNA gene; downstream from the sequence b) suitable restriction sites for the cloning of the sequence transcribing the pre-siRNA; downstream from these sites c) sequences derived from the sequences at 3' of the gene for U1 snRNA that are necessary and sufficient for the correct formation of 3' of the pre-siRNA.

WO 2005/005634 A2